Huntington's Disease Fact Sheet

CIRM funds many projects seeking to better understand Huntington's disease and to translate those discoveries into new therapies.

Description

In the U.S. about 30,000 people have been diagnosed with Huntington's Disease (HD) and another 150,000 have a 50 percent risk of developing the disease because they have one parent who has or had HD. There are no effective therapies and the disease is uniformly fatal, usually in 10 to 20 years.

Huntington's Disease results from a mutation to one gene, but that mutation can vary between patients and that may be linked to differences in how the disease progresses in various patients. But in all cases the mutated gene produces a protein that is toxic to nerve cells and eventually kills them. California's stem cell agency has funded several projects that probe into both the nature of the mutation and ways to prevent or repair the damage from the mutant protein (a full list is below).

One obstacle to finding and testing potential therapies for HD is the lack of a good laboratory model that captures the complexity of the disease in people. So researchers are using stem cells to create a "disease in a dish" model, that allows them to create cells that reflect different forms of Huntington's disease and then use them to screen different drugs to see if they are effective against HD. These teams are taking advantage of adult cell reprogramming to create so called induced Pluripotent Stem Cells (iPS) from patients with HD to see how the cells differ. One team, over time, also hopes to genetically modify these iPS cells so that they produce the correct Huntington protein and could be used as a personalized therapy, custom-designed for each person with HD, to reduce the chance their own immune system will destroy the new cells.

Two other CIRM-funded teams are seeking to identify potential therapies that could be delivered to the damaged nerves with stem cells. One proposes to use embryonic stem cells, mature them into early forms of those nerve cells and genetically modify them to deliver a compound that could protect patient's other nerves from the toxic protein. The other plans to use a type of stem cell found in bone marrow, mesenchymal stem cells, to deliver a genetic fragment called iRNA to the nerves and shut down the faulty gene.

Clinical Stage Programs

University of California, Davis

This research team plans to use bone marrow derived mesenchymal stem cells to deliver a growth factor to patients' damaged and endangered nerves. The factor they have chosen, called BDNF, has been shown to be effective in laboratory studies in reducing nerve cell death and improving the function of nerves. They completed an observational phase trial to monitor disease progression in a group of patients and have decided to conduct additional laboratory work with their cell-plus-gene candidate therapy.

- Read about the team's progress
- Read about the Observational Trial

CIRM Grants Targeting Huntington's Disease

Researcher name	Institution	Grant Title	Grant Type	Approved funds
Ali Brivanlou	Rumi Scientific CA	Discovery of therapeutics for Huntington's Disease	Quest - Discovery Stage Research Projects	$1,399,800
Leslie Thompson	University of California, Irvine	A hESc-based Development Candidate for Huntington's Disease	Early Translational II	$3,955,038
Leslie Thompson	University of California, Irvine	New Cell Lines for Huntington's Disease	New Cell Lines	$1,302,526
Jan Nolta	University of California, Davis	Sustained siRNA production from human MSC to treat Huntingtons Disease and other neurodegenerative disorders	Early Translational I	$2,615,674
Joel Gottesfeld	Scripps Research Institute	Triplet Repeat Instability in Human iPSCs	Basic Biology III	$1,705,494
Vicki Wheelock	University of California, Davis	MSC engineered to produce BDNF for the treatment of Huntington's disease	Disease Team Therapy Planning I	$97,564
Vicki Wheelock	University of California, Davis	MSC engineered to produce BDNF for the treatment of Huntington's disease	Disease Team Therapy Development - Research	$8,924,235
Clive Svendsen	Cedars-Sinai Medical Center	The HD iPSC Consortium: Repeat Length Dependent Phenotypes for Assay Development	iPSC Consortia Award	$300,000
Steven Finkbeiner	Gladstone Institutes	Common molecular mechanisms in neurodegenerative diseases using patient based iPSC neurons	Basic Biology IV	$1,482,025

John Griffin	Numerate, Inc.	Use of human iPSC-derived neurons from Huntington's Disease patients to develop novel, disease-modifying small molecule structural corrector drug candidates targeting the unique, neurotoxic conformation of mutant huntingtin	Early Translational IV	$520,015	
Leslie Thompson	University of California, Irvine	Huntington's Disease Team	Disease Team Planning	$41,953	
Leslie Thompson	University of California, Irvine	A hNSC Development Candidate for Huntington's Disease	Preclinical Development Awards	$4,951,623	
LESLIE Thompson	University of California, Irvine	HD-CARE INAUGURAL SYMPOSIUM	Conference II	$1,500	
Leslie Thompson	University of California, Irvine	hNSC-mediated delivery of ApiCCT1 as a candidate therapeutic for Huntington's disease	Quest - Discovery Stage Research Projects	$1,650,263	
					Total: $28,947,710.00

CIRM Huntington's Disease Videos

 Ningzhe Zhang, Buck Institute - CIRM Stem Cell #SciencePitch	 Leslie Thompson, UC Irvine - CIRM Stem Cell #SciencePitch	 Vicki Wheelock, UC Davis - CIRM Stem Cell #SciencePitch	 Huntington's Advocates Celebrate New Stem Cell Research Funding
 Judy Roberson: Patient advocates drive stem cell scientists	 Alzheimer's and Huntington's - Using Stem Cells to Understand and Treat Disease	 Disease in a Dish - Using Stem Cells to Model Huntington's Disease and SMA	 Huntington's Disease: Progress and Promise in Stem Cell Research
 Spotlight on Huntington's Disease (2010): Welcoming Remarks	 Spotlight on Huntington's Disease (2010): Vicki Wheelock	 Spotlight on Huntington's Disease (2010): Jan Nolta	 Spotlight on Huntington's Disease (2010): Sherry
 Spotlight on Huntington's Disease (2010): Closing Remarks	 Spotlight on Huntington's Disease (2007): Welcoming Remarks	 Spotlight on Huntington's Disease (2007): Seminar by Robert Pacifici, Ph.D.	 Spotlight on Huntington's Disease (2007): Seminar by Hans Keirstead, Ph.D.
 Spotlight on Huntington's Disease (2007): Seminar by Frances Saldana			

News and Information

- Stem Cells Show Promise For Treating Huntington's Disease (ScienceDaily)
- UC Davis researchers awarded new stem cell grants (UC Davis)
- Race against the clock (UC Irvine)
- Overview of stem cell research for Huntington's Disease (HDSA)

Resources

- NIH Huntington's Disease Information
- Find a clinical trial near you: NIH Clinical Trials database
- Huntington's Disease Society of America
- Family Caregiver Alliance
- National Family Caregivers Association
- The *Movement* Disorders Society

Find Out More:
Stem Cell FAQ | Stem Cell Videos | What We Fund

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